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STATEMENT

PRESCRIPTION DRUG USER FEE ACT (PDUFA)
PUBLIC MEETING
SEPTEMBER 15, 2000

BY

THE NATIONAL ORGANIZATION FOR RARE DISORDERS
ABBEY MEYERS, PRESIDENT

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ABBEY S. MEYERS
PRESIDENT

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We are pleased that patient advocacy groups such as the National Organization for Rare Disorders (NORD) have been invited to participate in discussions about the reauthorization of the Prescription Drug User Fee Act (PDUFA) of 1992. Being given this historic opportunity to impact the future of PDUFA is profoundly important because until today consumer and patient organizations have been excluded from the previous PDUFA I and II negotiations, generating an atmosphere of distrust and skepticism about the actual intent of the legislation.

While the goal of PDUFA was aimed at giving FDA adequate resources to speed the approval of new drugs to the American market, we question who the real beneficiaries actually are. Was the objective to help consumers gain speedier access to important therapeutic advancements, or was it aimed at helping the most profitable industry in the U.S. for the past 20 years? The perception among many patient and consumer groups is the later.

Granted, since the enactment of PDUFA, user fees have provided the FDA with the resources needed to process drug and biologic applications quicker than the agency was able to do in previous years, but we must doubt the laws' true intent. The legislation was passed to ensure rapid access to NEW and innovative therapies to treat debilitating and life-threatening diseases and disorders. Yet, according to the National Institute of Health Care Management, nearly one-half of the drugs approved by the FDA in the 1990's were for new formulations or new combinations of EXISTING drugs. And so, once again, we must ask – who are the true beneficiaries of the Prescription Drug User Fee Act – consumers or pharmaceutical companies?

In a perfect world the answer to this question would be the authorization of adequate federal funding for the ENTIRE agency and thus no economic need for user fees at all. However, realizing that in all probability such a remedy may not be politically practicable, we suggest that FDA implement user fees and set annual performance targets for other programs and products including medical devices, generic drugs, foods, and laboratory inspections, etc. This increased funding for the agency could then be used to support vital programs within the agency such as pharmaceutical marketing surveillance and adverse event monitoring. User fees, if renewed, SHOULD NOT BE RESTRICTED TO THE NEW DRUG APPROVAL PROCESS.

The public expects the FDA to be the primary consumer protection agency of the American

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Prescription Drugs and Intellectual Property Protection: Finding the Right Balance Between Access and Innovation, The National Institute for Health Care Management, August 2000.

government. But there is the conviction among many that the agency is far more responsive to the needs of pharmaceutical companies and not to the safety and welfare of the American consumer. PDUFA raises the specter of conflict-of-interest. Is the agency more responsive to pharmaceutical companies because they are the primary customers of the FDA? To many consumer and patient groups, this appears to be the case.

Is there a way to alter this perception without sacrificing consumers speedy access to life saving drugs? If PDUFA is to be reauthorized, we believe the answer is yes. There are solutions.

1. The agency must prioritize speedy reviews so that significant therapeutic advancements for serious and life threatening diseases are reviewed quicker than products that have little or no therapeutic superiority over existing drugs, or medicines for currently treatable health conditions.

The approval of products that do not treat serious or chronic diseases – for example, lifestyle drugs such as the recently approved *Vaniqua*, a topical application for the removal of facial hair, or the blockbuster *Viagra*, a treatment for erectile dysfunction – should not be approved over innovative drugs that reduce suffering, promote healing and improve health. Important new life-saving therapies such anti-cancer agents or an enzyme replacement therapy for a genetic disease must take precedence over the third or fourth cox-2 inhibitor or any other me-too or lifestyle drug.

Under current FDA policy, the public health importance of each potential new drug does not seem to be a major factor in choosing which products will be rushed to market, and which will require more intensive review. I would like to suggest, therefore, that consideration of priority applications be reviewed in some manner by a committee that includes consumer representation to ensure that truly innovative therapies come to market prior to me-too and lifestyle drugs.

2. PDUFA should not be restricted to new product reviews.

As I mentioned just a few moments ago, the ideal alternative to PDUFA III would be adequate federal funding for the entire agency appropriated by Congress every year rather than PDUFA III. However, if the Act is to be reauthorized, the FDA should not be forced to focus its resources on the approval of new drugs and biologics only.

The FDA is a consumer protection agency, not a new drug development agency, and so it must protect and serve the interests of the public's health beyond the NDA process. Other agencies and departments within the FDA are currently understaffed and overworked simply because PDUFA has drained the resources needed to adequately complete critically important public health responsibilities.

User fees should be implemented for other programs and products including medical devices, generic drugs and laboratory inspections, etc., as well as the policing of advertising enforcement and adverse event monitoring. Like the performance goals established under the current PDUFA, flexible objectives and targets should be implemented across-the-board.

The agency's success should be based on the completeness of its work to ensure that safe and

effective therapies are approved, rather than set inflexible performance goals based solely on stringently defined timeframes.

3. FDA should reform its "gag rule" to allow FDA personnel to honestly respond to consumer inquiries about delays in new drug approvals.

Currently the FDA considers most information about investigational products to be "trade secrets." As a result, there is often political pressure applied on the agency to approve specific drugs even though the agency knows there are significant problems with the product. Because the FDA is not permitted to explain its delays to the public, it leaves itself open to intense criticism and consumers are left in the dark. FDA must have the authority to respond truthfully to consumers' questions about investigational drugs.

4. User fees should be waived under specific and limited circumstances.

There are many start-up companies, including orphan drug manufacturers and humanitarian device companies, that are not yet profitable, and have limited resources available to them to bring important therapeutic discoveries to market. Accommodations should be made to waive user fees for the product, facility and application fees for eligible start-up and not-yet-profitable companies under very specific and stringent circumstances. Protections must also be instituted to ensure that the larger pharmaceutical companies do not circumvent current law and take advantage of the waiver system to the detriment of the agency and its programs. The agency can not be perceived as favoring some companies and products over others simply because more money changes hands.

Conclusion

While adoption of these proposals will be an important first step to resolve the problems and concerns we have about the user fee program, there are a number of significant shortcomings the FDA must take immediate steps to resolve.

For example, the performance goals of the current PDUFA legislation are measured in time without adequate safeguards in place to quantify safety and effectiveness. There have been too many recent withdrawals of marketed drugs that have maimed and killed people. Although the FDA argues that "it has made the drug and biologics review process more efficient without lowering drug review standards," we suspect that the large number of market withdrawals since PDUFA was implemented tends to indicate that increased speed of NDA and PLA reviews may not be the wisest public health policy.

No matter how fervently the FDA may continue to argue that the systems in place guarantee the safety and welfare of American consumers, the fact remains that it is "perceived" that the public's best interests are not being considered.

Second, the FDA and Congress must not take the "one size fits" all approach to drug approval. It is wrong to require the agency to spend the same amount of time it takes to review a

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User Fees for Faster Drug Reviews: Are They Helping or Hurting the Public Health, FDA Consumer Magazine, September-October 2000.

promising drug that will save lives, with me-too and lifestyle drugs. People are suffering and dying every day from horrific diseases. But to our knowledge no one has yet died from an excess of facial hair or erectile dysfunction.

And third, since the 1980's the FDA has had adequate tools in place to enable patients to obtain drugs quickly and before they are approved for marketing (the Treatment IND research phase) thus providing desperately ill patients access to potentially important medicines. There is absolutely no public health benefit to rushing me-too anti-inflammatories or anti-hypertensives through the approval system.

Again, thank you for allowing patient and consumer organizations to participate in this important meeting. The National Organization for Rare Disorders (NORD) looks forward to additional opportunities to impact these negotiations.